

IMB TechTalk

Dr. Rainer Ebel

Sigma-Aldrich

"Targeted Genome Editing in Mammalian Cells Using Engineered Zinc Finger Nucleases"

Tuesday, November 15th, 2011, 11:00 h (*s.t.*)

Venue: 2nd Floor Seminar Room Institute of Molecular Biology (IMB) Johannes Gutenberg University Campus Mainz

All are welcome to attend

Host: Dr. Bernhard Korn, Director CF & Technology, IMB For further information, please contact: Bianca Steinbach, E-mail: <u>b.steinbach@imb-mainz.de</u>, tel.: +49-(6131)-39-21510



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Speaker: Dr. Rainer Ebel

Title: Targeted Genome Editing in Mammalian Cells Using Engineered Zinc Finger Nucleases

Abstract: Rational genome engineering in mammalian cells is of enormous potential across basic research, drug-discovery as well as cell-based medicines. Zinc finger nucleases (ZFNs) are a class of engineered DNA binding proteins that facilitate targeted editing of the genome by creating double- strand breaks at user-specified locations.

Within these chimeric proteins the DNA binding specificity of the zinc finger protein determines the site of nuclease action. Such engineered ZFNs are able to recognize and bind to a specified locus and evoke a double-strand break in the targeted DNA with high efficiency and base-pair precision. The cell then employs the natural DNA repair processes of either homology-directed repair or non-homologous end joining to heal the targeted break.

These two pathways provide the investigator with the ability to provoke three unique outcomes in genome editing – gene correction, gene deletion and targeted gene addition. Furthermore, the speed and efficiency of this process enables us to knockout multiple genes in the same cell.

Drawing from our work with transformed cell lines, primary human cells, and multipotent stem cells, we will present examples of single, double and triple gene knockout, as well as targeted gene insertion into native chromosomal loci. We will also discuss recent work that shows the effectiveness of ZFN-mediated gene knockout in embryos for the creation of animal models such as rat.

Links to publications:

mouse: "<u>http://www.ncbi.nlm.nih.gov/pubmed/21151125</u>" zebra fish: "<u>http://www.ncbi.nlm.nih.gov/pubmed/18500334</u>" stem cells: "<u>http://www.ncbi.nlm.nih.gov/pubmed/19680244</u>"